Dystrophin minigene constructs

Human dystrophin coding sequence 11058bp

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| 33 H2 R4 R5 R6 R7 R8 F | |
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| 1 R2 R3 H2 R4 R5 R6 R7 R8 R9 R10 R11 R12 R13 R14 R15 R16 R17 R18 R | |
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| N H/ R/ R2 R3 R22 R23 R24 H4 CR | Δ4173 | |
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| N H/I R/I R2 H3 R22 R23 R24 H/4 CR | Ψ3990 | |
| N H1 R1 R2 R22 R23 R24 H4 CR | Λ3849 | |
| N H1 R1 R22 R23 R24 H4 CR | Δ3531 | |
| N H4 R1 R2 R23 R24 H4 CR | Δ3510 | |
| N HI RI R2 R3 R24 H4 CR | Δ3447 | |
| N MI RI R24 H4 CR | Λ2796 | |
| N HI RI HA CR CT | DysM3 (3700bp) | |
| TTR MCK Promoter Dystrophin minigene | gene PolyA ITR | AAV-MCK-mini-dystrophin |
| TR CMV Promoter Dystrophin minigene | ene PolyA ITR | AAV-CMV-mini-dystrophin |
| | FIG. 1 | |



mdx+∆3990

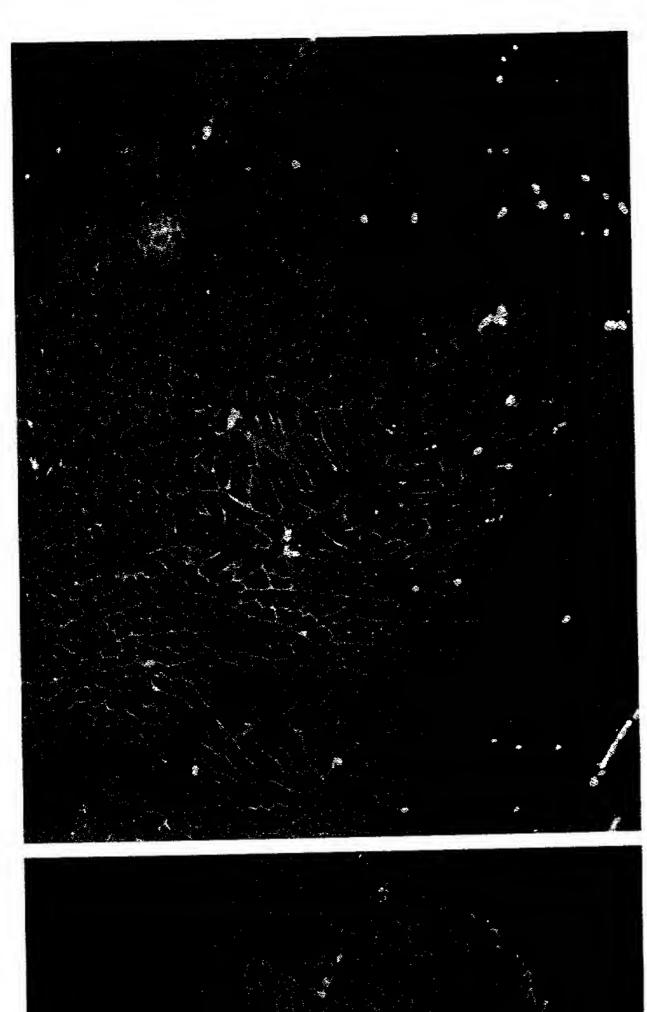
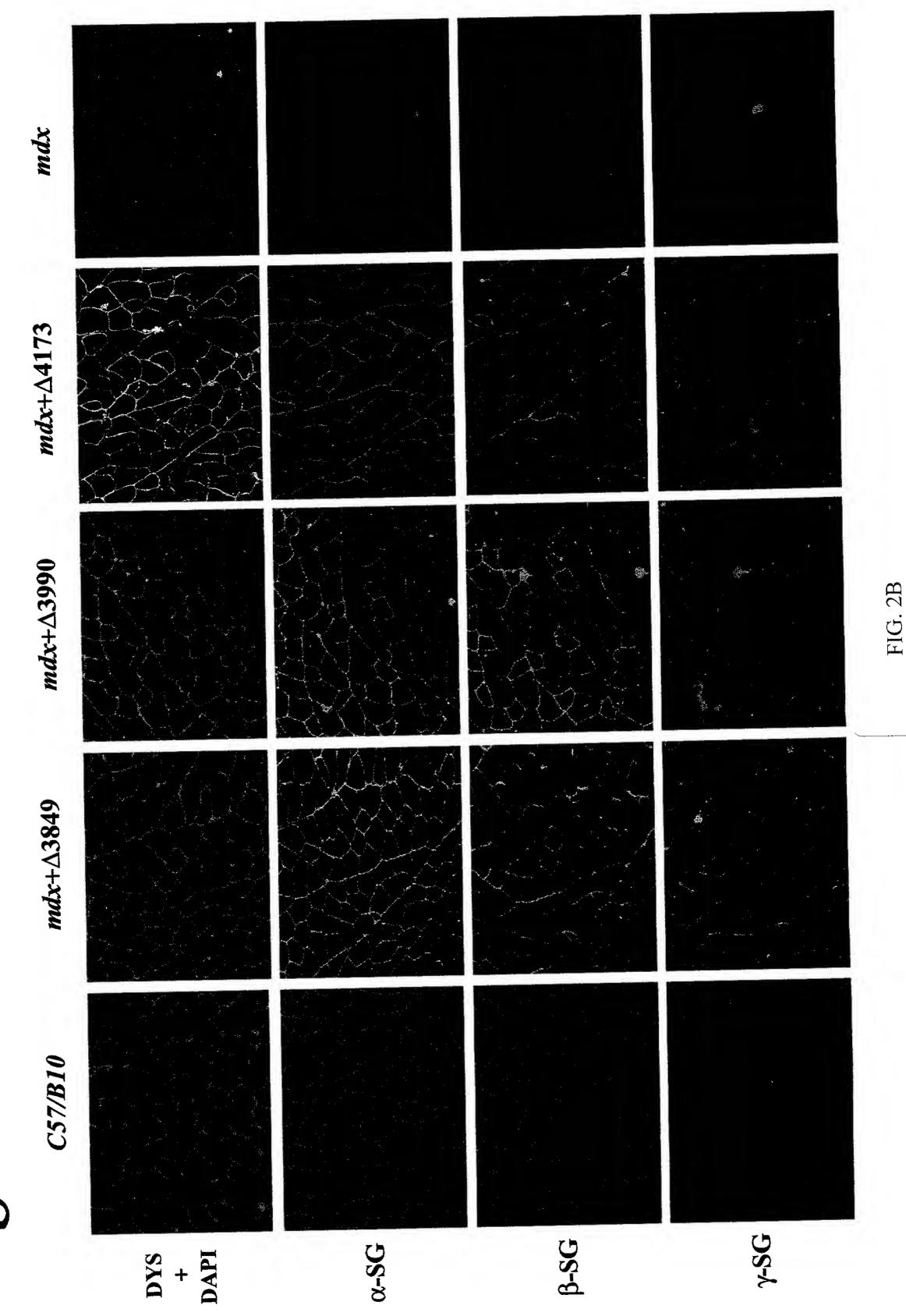


FIG. 2A



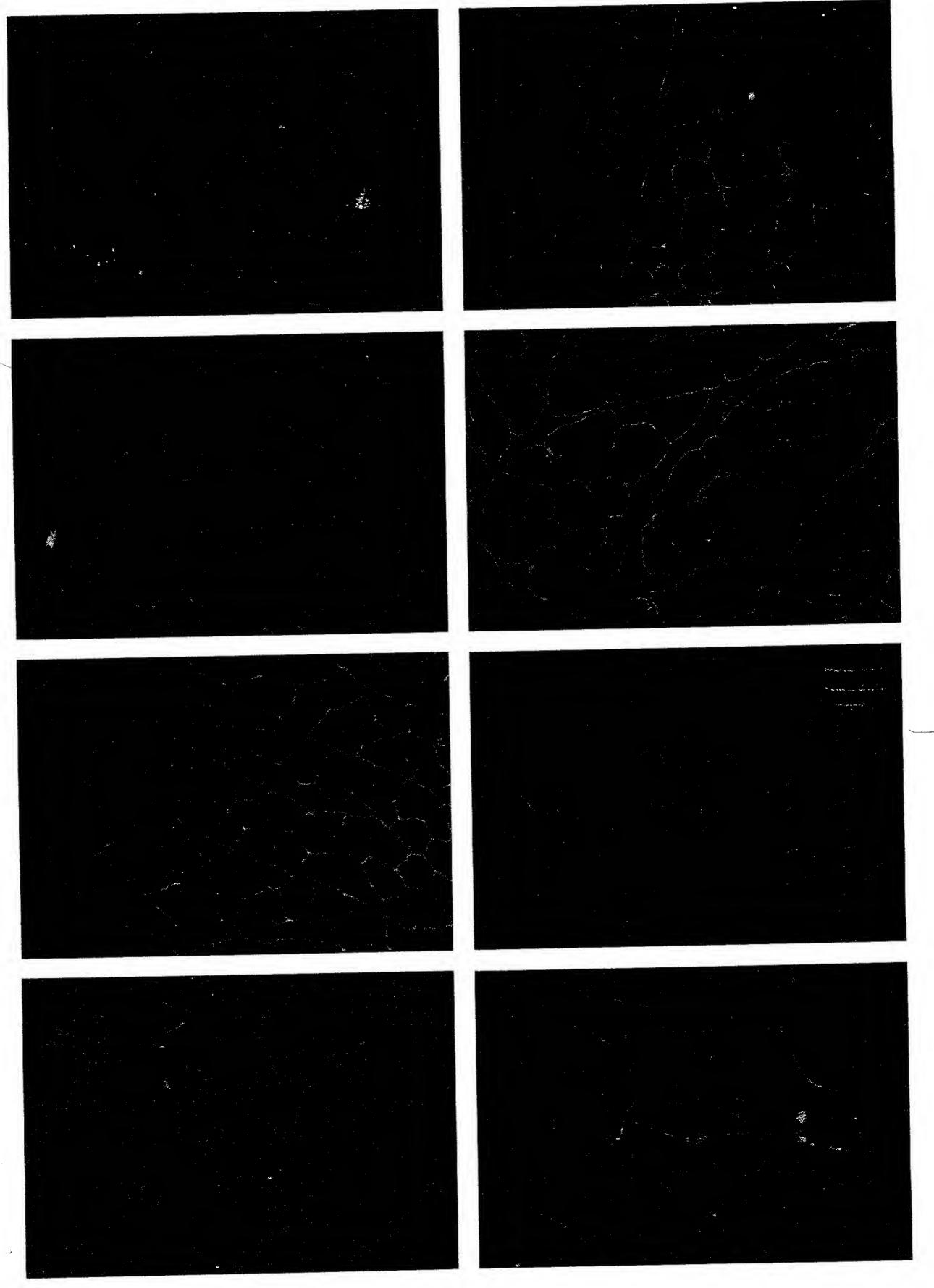
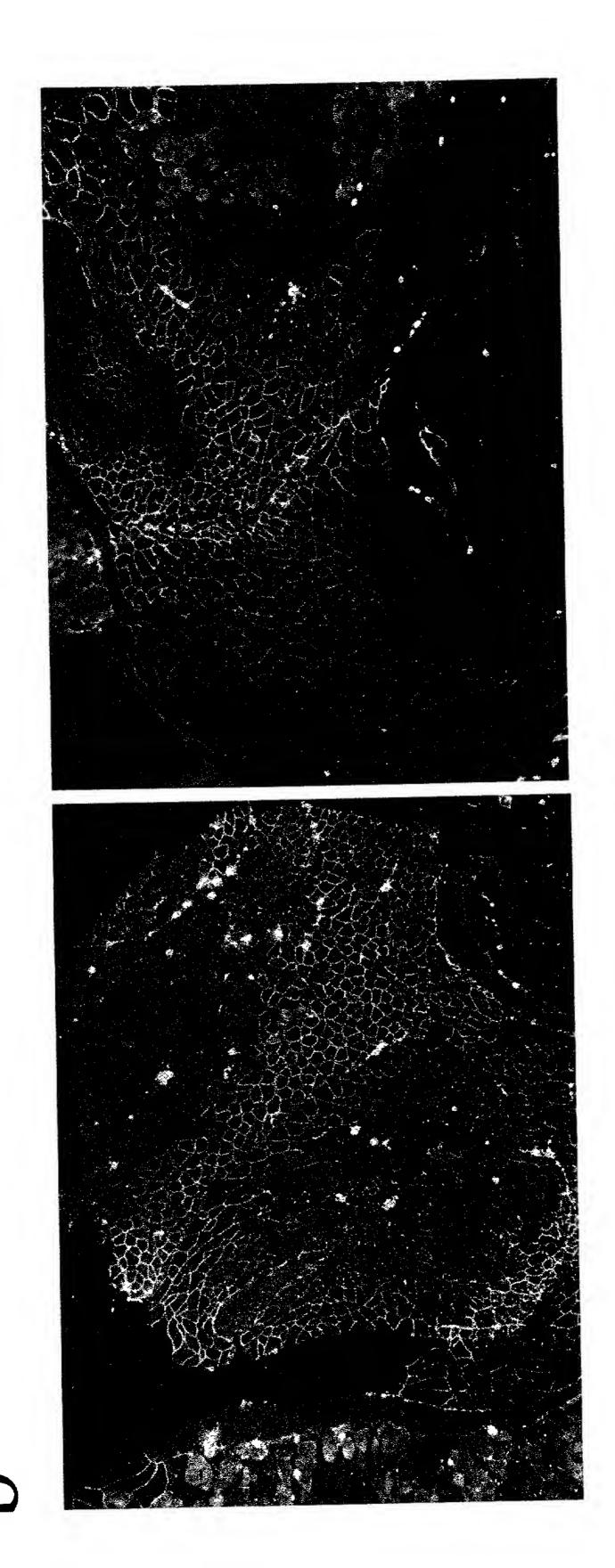


FIG. 3

FIG 4A



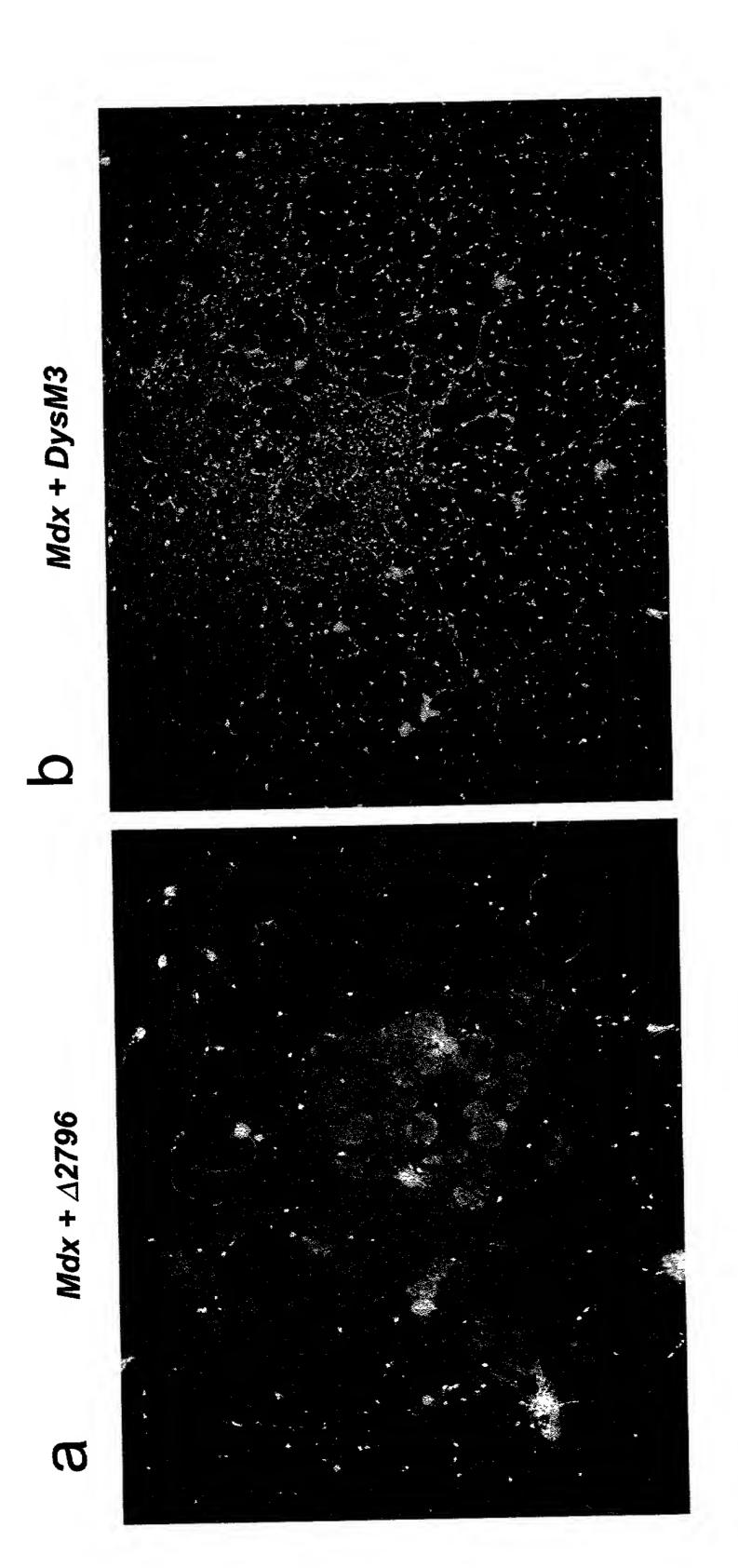


FIG. 5

Lengthening Activation

